UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

IN RE PTC THERAPEUTICS, INC. SECURITIES LITIGATION	Civ. No. 16-1124 (KM) (MAH) **OPINION**

KEVIN MCNULTY, U.S.D.J.:

The plaintiffs brought this securities class action against PTC Therapeutics, its CEO and founder, Dr. Stuart Peltz, and its CFO, Shane Kovacs (collectively, "defendants" or "PTC"), after the FDA found PTC's New Drug Application ("NDA") for Translarna facially inadequate for review. Plaintiffs, who purchased PTC stock sometime between November 6, 2014 and February 23, 2016, allege that PTC misrepresented or omitted facts about the efficacy of Translarna while knowing all along that the clinical data failed to meet FDA approval standards. As a result, plaintiffs claim, they suffered substantial financial losses when PTC's share price plummeted nearly 60% following the public announcement of the FDA's refusal to file its NDA. Defendants have moved to dismiss the complaint, claiming that plaintiffs fail to allege any actionable misstatement or omission made with intent to defraud or deceive. The motion to dismiss will be granted in part and denied in part.

I. FACTUAL BACKGROUND[1]

A. PTC, Translarna, and DMD

Founded in 1998 by Dr. Peltz, PTC develops drugs that treat rare and ultra-rare genetic diseases and disorders. In 2003, PTC began developing ataluren (brand name Translarna), which it designed to treat a genetic mutation called a "nonsense mutation." Nonsense mutations can cause a variety of serious genetic diseases, including a particularly rare and devastating disease known as Duchene muscular dystrophy ("DMD").[2] With a process called "post-transcriptional control," PTC hoped that that Translarna would slow the disease's progress by allowing cells to "read-through" the nonsense mutation and produce functional proteins. (AC ¶¶ 2, 30, 34, 35, 38-39)

Translarna was the first product for which PTC sought regulatory approval in the United States and Europe. Because PTC's other drugs were years away from being marketed, Translarna was PTC's only opportunity to begin generating revenue during the class period. The approval of Translarna, if obtained, would reflect favorably on theory of post-transcriptional control, which might assist PTC in marketing and developing other drugs that treat disorders caused by nonsense mutations. Translarna accounted for 100% of PTC's revenues in February 2016. (AC ¶¶ 34-37, 184)

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1 Citations to the record are as follows:

"AC" — Consolidated Amended Class Action Complaint, ECF No. 52

"Def. Ex. __" — Exhibits attached to the Declaration of Deborah S. Birnbach, Esq., filed February 14, 2014, ECF No. 56-2

2 DMD, which predominantly occurs in boys, causes the muscles to weaken and progressively waste away. Patients typically exhibit symptoms at two or three years old, and by adolescence are unable to walk. When DMD eventually reaches the lungs, a respirator is required; when it reaches the heart, the patient dies.

B. The Drug Approval Process: An Overview

A company, such as PTC, which wants to market and sell a new drug, such as Translarna, must submit a new drug application ("NDA") to the FDA. To approve the drug, the FDA must be convinced that there is "substantial evidence" that the drug is safe and effective at treating the condition it purports to treat. The developer usually does this by conducting a series of clinical trials. The first, a Phase 1 trial, evaluates the drug's safety and dosage tolerance. The second, a Phase 2 trial, evaluates safety, dosage, and efficacy. Phase 2 is sometimes broken up into two sub-phases, 2a and 2b. As a rule of thumb, Phase 2b trials are more intensive than Phase 2a trials; they evaluate a drug's efficacy in a larger patient population or over a longer period of time than a 2a trial, and as against a placebo. A phase 3 trial, usually the final trial, also evaluates safety and efficacy, but in an even larger patient population. To secure an NDA, the FDA typically requires two successful efficacy trials, ideally a phase 2b and a phase 3 trial. (*Id.* ¶¶ 40-49)

Once an NDA is submitted, the FDA conducts a preliminary review of the application. If it is incomplete, improperly constructed, or otherwise facially inadequate, the FDA will issue a RTF—Refuse to File—letter. According to the FDA:

> A RTF is based on omission of clearly necessary information . . . or omissions or inadequacies so severe as to render the application incomplete on its face and where the omissions or inadequacies are so obvious, at least once identified, and not a matter of interpretation or judgment about the meaning of data submitted."

(AC ¶¶ 50-52)

RTFs, plaintiffs say, are relatively uncommon. From January 2010 to February 2016, the FDA issued 16 RTFs out of more than 200 NDAs for new molecular entities (not including the two Translarna RTFs).[3] (*Id.* ¶¶ 53-54)

Because DMD is so rare and so serious, Translarna benefited from two programs during the development and review process. From the FDA's Orphan Products Clinical Trial Grants Program, PTC received a grant to help fund Translarna's clinical trials. The FDA also designated Translarna as a "fast track" drug, which allowed PTC to submit Translarna's NDA on a rolling basis, instead of waiting until after all the trials had been completed. The FDA encourages fast-track drug developers to communicate with it "early" and "frequent[ly]" during the development and review process. (*Id.* ¶¶ 40-43)

C. Translarna's Development and Review

1. The 2011 2b Trial

By May 2007, PTC had completed the Phase 1 and Phase 2a trials for Translarna. Patients began enrolling in the 2b trial—Translarna's first major efficacy trial—in February 2008. 174 DMD patients between the ages of 5 and 20 enrolled. (*Id.* ¶ 55)

The goal of the 48-week 2b trial was to determine whether Translarna-treated patients experienced a slower decline in their ability to use their muscles. To test that hypothesis, one group of patients was given Translarna while a second group was given a placebo. At the beginning (week one) and end (week 48) of the trial, the researchers measured the distance that each patient could walk in six minutes. The difference between those two figures was then calculated, and averaged across the relevant group. If the average change in distance walked by Translarna-takers exceeded that of placebo-takers by more

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[3] As plaintiffs acknowledge, the FDA does not disclose which drugs receive RTFs, and a publicly traded company (like PTC) need only disclose the existence of an RTF (and even then, not necessarily its contents) if it considers the RTF to be a material event. The data, then, may be incomplete. (AC ¶ 53)

than 30 meters, then PTC could conclude that Translarna had a clinically meaningful effect on DMD patients. To achieve statistical significance, the results would need a p-value of 0.05 or less.[4] (AC ¶¶ 56-59)

In December 2009, the 2b trial wrapped up. On March 3, 2011, PTC released some preliminary results. Translarna failed to meet the pre-specified endpoints for effectiveness and statistical significance. At 29.7 meters, the mean change in 6-minute walking distance fell just short of 30-meter threshold necessary to demonstrate clinical effectiveness, and the p-value for the results, 0.149, far exceeded the .05 limit of statistical significance. PTC theorized that the 2b trial failed to reach its endpoints because the study included "younger patients and patients with higher baseline 6-minute walk distances [that] are less likely to exhibit measurable declines in 6-minute walk distance over 48 weeks." The problem, in other words, was that 2b trial included patients that were not yet sick enough to report a benefit—as PTC had defined it—from Translarna. PTC therefore removed the younger, more able patients from the dataset, and performed a retrospective data analysis on the older, "decline-phase" patients. These so-called "corrected" results were better, and met the p-test of statistical significance. (AC ¶¶ 60-63)

In March 2011, PTC filed an NDA (the "2011 NDA") for Translarna based on the corrected findings. Because "Phase 2b clinical trial contained in the NDA did not achieve statistical significance in the pre-specified analysis," the FDA refused to file the NDA (the "2011 RTF"). PTC appealed in December 2011, and the FDA affirmed its decision in February 2012. (*Id.* ¶¶ 64-66)

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[4] The p-value for a dataset represents the probability that the hypothesis being tested was borne out simply by chance. In lay terms, a p-value of 0.05 means that there is a 5% likelihood that an occurrence was the result of chance alone. This is sometimes referred to as statistical significance, a term of art; it does not imply that results are "significant" or important as those words are used colloquially.

In August 2014, the European Medicines Agency ("EMA") authorized PTC to market Translarna in Germany.[5] Although the EMA initially had "major

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[5] Defendants have submitted a slew of extrinsic documents with their papers, a number of which plaintiffs have moved to strike. (ECF Nos. 56, 58) Many are clearly subject to judicial notice (*i.e.*, PTC's 10-Q's, 8-Ks, transcripts of conference calls relied upon in the AC, etc.), and plaintiffs do not contend otherwise. *See generally* Fed. R. Evid. 201 ("The court may judicially notice a fact that is not subject to reasonable dispute because it: (1) is generally known within the trial court's territorial jurisdiction; or (2) can be accurately and readily determined from sources whose accuracy cannot reasonably be questioned.") Because these documents are publicly available, authentic records, and are explicitly relied upon and integral to the complaint, I will take judicial notice of them. *See In re NACH Sec. Litig.*, No. CIV.A 00-4020, 2001 WL 1241007, at *5 (E.D. Pa. Oct. 17, 201), *aff'd* 306 F.3d 1314 (3d Cir. 2002).

I will decline to take judicial notice of other documents, however. Defendants have submitted a number of analyst reports (Def. Exs. 28-31), SEC filings of its competitors, (Def. Exs. 34-35), and their own 8-K and 10-Q, filed after the close of the class period. (Def. Exs. 19-20). The AC relies upon none of these documents. *See In re Asbestos Product Liability Litig. (No. VI)*, 822 F.3d 125, 134 n.7 (3d Cir. 2016) (on a motion to dismiss, a courts may consider "document[s] integral to or explicitly relied upon in the complaint" or any "undisputedly authentic document that a defendant attaches as an exhibit to a motion to dismiss if the plaintiff's claims are based on the document") (internal citations omitted)).

Defendants observe that I have the discretion to notice these documents, not for their truth, but for the existence of their contents. Some courts, for example, have taken notice of analyst reports and SEC documents not relied upon in the complaint. *NAHC*, 306 F.3d at 1331; *SEC v. Ustain*, 229 F. Supp. 3d 739, 761 (N.D. Ill. 2017). Defendants cite no authority, however, for the proposition that a court may take notice of the SEC filings of *another company* to resolve a motion to dismiss a securities lawsuit. At any rate, the inferences defendants wish to draw from these documents go beyond the mere existence of statements within them (*e.g.*, what PTC actually believed about the ACT DMD results, what the investing public actually believed about the ACT DMD results, etc.). Such contentions cannot properly be weighed on a motion to dismiss. That is all the more true since materiality is not an issue on this motion. *Ustain*, 229 F. Supp. 3d at 761 (taking judicial notice of analyst reports "to resolve questions about the materiality of alleged misrepresentations or omissions").

None of these documents would change my analysis in any event. Defendants submit the analyst reports to demonstrate that the market was generally aware that the ACT DMD study had failed to reach its endpoints. But those reports also reiterate the allegedly false or misleading information that management conveyed to the market by other means: in particular, that the 300-400 meter subgroup results, standing alone, could be sufficient to for FDA approval. As explained in Part II.B.1-2, it is sufficiently alleged that those assertions were false or misleading.

PTC proffers the SEC filings of its competitors to prove up the point that the FDA had filed competitors' NDAs even though their clinical trials had failed to meet

objections" because it believed there was "insufficient evidence of efficacy based on [PTC]'s single Phase 2b clinical trial," it was eventually convinced by the post-hoc analysis of the data described above. The EMA authorization was "conditioned upon the successful completion of ACT DMD and subject to annual review and renewal by the EMA." (Def. Ex. 6, pp. 3, 4, 14; Def Ex. 10 pp. 3-4, 6)

2. The 2015 ACT DMD (Phase 3) Trial

Following the 2011 RTF, PTC pushed onward with the development and design for the phase 3 trial, also known as the Ataluren Confirmatory Trial in DMD, or "ACT DMD." The goal of the ACT DMD trial would be to "confirm" the positive results from the older, decline-phase patients that seemed to benefit most from Translarna in the 2b trial. Enrollment in the ACT DMD trial was therefore restricted DMD patients from ages 7 to 16 who could still walk. (AC ¶¶ 72-74)

As far as structure and design, the ACT DMD study was essentially the same as the 2b study: a 48-week, double blind trial measuring six minute

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their primary endpoints or weren't complete at all. What PTC believed about other company's disclosures is several steps removed from the issues presented here. To the extent those filings are relevant at all, I could not give them weight in this procedural posture.

PTC's own November 2016 and January 2017 SEC filings are offered to substantiate two relatively inconsequential contentions: After the FDA issued Translarna its second RTF, PTC decided to file an NDA "over protest," and the EMA renewed Translarna's marketing approval. Each of those events occurred nearly a year after the allegedly false or misleading statements, however, and so their relevance to the issues here—*e.g.*, what was known to PTC at the time it made the alleged misstatements—is quite low.

The last item plaintiffs wish to strike is a 16-page summary chart (Def. Ex. 2). The chart compares the alleged misstatements side-by-side with defendants' arguments as to why those statements are not false or misleading. Plaintiffs argue that the chart is not the proper subject of judicial notice. Their real gripe seems to be that defendants effectively augmented the size of their opposition brief by attaching the chart as an exhibit. Plaintiffs do not argue, however, that they are prejudiced in any way by the chart. Nor can I discern prejudice: The chart does nothing more than organize portions of the AC (reprinted verbatim) and refer to arguments already raised in the papers. Although I assign it little significance, I also decline to strike it.

walking distance, a 30-meter clinical benefit endpoint, a .05 or less statistical significance threshold, and so on. After the study concluded, PTC planned to perform a "meta-analysis" combining all of the ACT DMD data with the favorable 2b study decline-phase data. (AC ¶¶ 71-77)

PTC would also take a closer look at two subgroups that it had pre-specified for statistical analysis. The first was a subgroup of patients who could not walk more than 350 meters at the beginning of the trial. PTC considered this subgroup "key" because "350 meters represents a transition point for patients towards a more rapid decline in walking ability as supported by analysis from our Phase 2b study." These children and teenagers, in other words, were the decline-phase patients around which PTC designed the entire ACT DMD study. The second pre-specified "key" subgroup consisted of patients who had a baseline that fell somewhere between 300-400 meters. This second group was specified "based on an increasing understanding of the sensitivity limitations of the six minute walk test as an endpoint in 48-week studies." That PTC had pre-specified either subgroup was not known publicly until after the ACT DMD results were announced. (AC ¶¶ 73-74, 77-82, 87)

During the development of ACT DMD trial, PTC told investors that its design reflected what it had learned from the 2b trial and incorporated the FDA's feedback, which made PTC confident that the study would succeed. Thus, for example, in an August 2013 earnings call, Peltz stated that "[t]he design of the trial reflects the knowledge gained from our earlier study as well as the views expressed in discussions with the FDA. . . . " A couple years later, in January 2015, Peltz stated at healthcare conference that PTC "used the learnings from our previous study to really wring out the risk in the current study." A few months after that, in May 2015, Kovacs, PTC's CFO, told attendees at a healthcare conference that PTC had "refined" the ACT DMD study "versus the prior Phase 2 study" and "had a high degree of confidence in

the likelihood of a positive outcome in this study later this year." (*Id.* ¶¶ 67-69, 114, 124)[6]

On October 15, 2015, PTC announced the ACT DMD results. They were worse than the 2b trial results. For the overall population of patients (sometimes referred as the overall "intent-to-treat," or "ITT" population), the mean change from baseline in the 6-minute walk test fell significantly below the 30-meter efficacy goal (15 meters) and well above the of .05 statistical significance threshold (p=0.213). But there was a bright spot: the 300-400 meter subgroup *did* report promising, statistically significant results. (47 meters, p=0.007). Corroborating those favorable results, PTC claimed, was the "pre-specified meta-analysis of their Phase 2b and ACT DMD results," although that analysis did not demonstrate meaningful clinical benefit. PTC did not disclose the <350 meter subgroup results.[7] (AC ¶¶ 81-84; Def. Ex. 13 p. 1-2)

In a Q&A conference call announcing the ACT DMD results, PTC emphasized the 300-400 meter subgroup and the pre-specified meta-analysis. PTC did not state that nearly 60% of the ACT DMD patients reported no clinically meaningful or statistically significant benefit from Translarna. The "totality of the data," PTC claimed, "confirmed" the clinical benefit of Translarna. As Peltz explained:

> The totality of the data for Translarna demonstrates clinical benefit across primary and secondary endpoints. We have pre-specified the key subgroup for analysis and the meta-analysis, both of which show Translarna had a clinically meaningful benefit for DMD patients. The results from ACT DMD trial showed consistent evidence of the clinical benefit of Translarna for individuals with nonsense mutation Duchenne muscular dystrophy, and its impact on the course

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[6] It is not clear when the ACT DMD trial actually started, although Peltz told investors that PTC had submitted the ACT DMD's proposed statistical analysis plan sometime in "spring 2015." (*Id.* ¶ 103)

[7] So far as this record reveals, PTC has never released the <350 subgroup results.

> of the disorder, and the quality of life for those boys and young men.

(AC ¶¶ 88-90, 128)

On the same call, Peltz implied that pre-specified meta-analysis combining the ACT DMD data with the decline-phase 2b trial data had the FDA's blessing:

> Q: Hi, thanks for taking the question. So have you had discussions with the FDA on the degree of consideration they might give to pre-specified meta-analysis? And if so, can you provide any more information on that?
>
> A. Sure, yes. Thanks for that question. The pre-specified meta-analysis was in our statistical plan, which we had discussions with with [sic] the FDA. This was in part, part of the pre-specified plan. So they were well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure.

More generally, Peltz told analysts that PTC's "approach was consistent with the recent draft guidance for Duchenne muscular dystrophy, but to pre-specify subgroups were a treatment effect is more likely to be seen, for the chosen primary and secondary endpoints." (AC ¶ 129; Def. Ex 23 p. 4)

About a month later, in a November 9, 2015, 8-K, 10-Q, and during a quarterly earnings call, PTC continued to represent that the "totality" of the data "confirmed" the clinical benefit of Translarna. From the 8-K:

> ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy. . . . The totality of the clinical data from two large, placebo-controlled clinical trials across 400 patients demonstrates Translarna's ability to slow disease progression.

(*Id.* ¶ 136)

From the 10-Q:

> [W]e believe that the results of the ACT DMD and the totality of clinical data across our two large, randomized placebo-controlled trials (ACT DMD study and our prior Phase 2b study, Study 007), provide substantial evidence of the

> effectiveness of Translarna and demonstrate a meaningful benefit of Translarna for the treatment of nmDMD.

(*Id.* ¶ 137)

And from the conference call (Peltz is speaking):

> [T]he goal is to show efficacy with given endpoints in the limited window of a 48 week clinical study. We see this in ACT DMD . . . the totality of clinical data confirmed Translarna's ability to slow disease progression for patients with DMD.

(*Id.* ¶ 138)

A week-and-a-half after that, on November 18, Kovacs spoke at a healthcare conference. He too implied that the "totality" and "consistency" of the data favored FDA approval:

> And the big picture about our data is and what will be part of our argument to both the regulatory authorities in the US and Europe is that the consistency of the results now seen across two of the largest placebo-controlled Phase 3 studies ever done in the disease, the totality of the data support the clinical benefit and certainly the risk-benefit profile of the drug in favor of an approval and getting something to these kids.

(AC ¶ 143)

In December 2015, Kovacs spoke at another healthcare conference. He told attendees that PTC's "intention today is for filing for full approval on the basis of two large well-controlled studies that all point to safety and efficacy for a risk-benefit profile in favor of the drug." (*Id.* ¶147)

3. The 2016 RTF

In January 2016, PTC announced that it had submitted a second NDA (the "2016 NDA") for Translarna. This NDA was for full, not conditional, approval; that is, PTC sought approval of Translarna for the treatment of *all* DMD patients, not just for patients of a certain age or at a particular stage of the disease. The 2016 NDA relied on (1) the 300-400 meter subgroup findings and (2) the meta-analysis combining the ACT DMD data with the decline-phase 2b trial data. PTC also submitted (3) a meta-analysis for *all* patients (*i.e.*, everyone in the ACT DMD ITT and "corrected" 2b patient populations) who had a baseline 6 minute walking distance of 300 to 400 meters. That analysis was not specified in advance of the either the ACT DMD or 2b trials; it was a post hoc analysis. It also accounted for less than 42% of all patients across both trials.[8] After receiving the ACT DMD results but before submitting the 2016 NDA, PTC did not meet with the FDA. (*Id.* ¶¶ 55, 92-93, 105, Def. Ex. 24)

While the FDA reviewed Translarna's NDA application, PTC continued to state or imply that substantial evidence supported approval. At yet another healthcare conference, this one held on January 13, 2016, Peltz stated:

> So you see in the two large studies where we used the six-minute walk test as the primary endpoint, we saw a benefit in the primary endpoint as well as the secondary endpoint. And in prespecified subgroups, we saw more robust effects being observed, both the primary and second endpoints. So consistent data in two independent studies.
>
> One of the things we've noticed they [presumably the FDA] asked for was sensitivity analysis, and that while you have prespecified subgroups, if you go beyond those, does the data still show clinically meaningful differences? And it does both in the primary and secondary endpoints.

(AC ¶ 150)

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[8] If all 174 patients that enrolled in the 2b trial (*i.e.*, the non-"corrected" population) are included in the calculation, that figure drops to less than 36%. (*See* Def. Ex. 24)

Discussing one of the meta-analyses, Peltz added:

> In the meta-analysis, where you combine the results, you see both in the six-minute walk distance as well as the time function tests, you see clinically meaningful and statistically significant improvements with Translarna over placebo. . . .
>
> And really it's consistent with totality of the data, demonstrating that this drug was efficacious. So I think we've checked that box.

(AC ¶ 151)

On February 22, 2016, the FDA issued Translarna a second RTF (the "2016 RTF"). Like the 2011 RTF, it is not publicly available. PTC relayed to investors the gist of it: The 2016 NDA was "not sufficiently complete to permit substantive review." "There were really two bases . . . that were outlined in the letter[,]" PTC said: "the first of which was that both the Phase 2b and Phase 3 studies had failed and therefore did not demonstrate substantial evidence of effectiveness and secondly that the application did not sufficiently describe the abuse potential of the drug." (*Id.* ¶¶ 96, 157)

After the announcement, the market turned on Translarna. From February 22 to February 23, 2016, PTC's share price fell from $28.26 to $10.84—a 61.6% drop. (*Id.* ¶¶ 158)

More details about the FDA's reasoning trickled out a week later. In a February 29, 2015 press release, PTC stated that the FDA viewed "certain of the company's adjustments to the ACT DMD study as post hoc and therefore not supportive of effectiveness." On a conference call the same day, an analyst asked Peltz to reconcile the FDA's position with PTC's previous representations "that the [ACT DMD] statistics plan was submitted to the FDA earlier in 2015." Peltz responded that PTC indeed had submitted the plan "in the spring of 2015." While the "FDA commented on our statistical analysis plan," he explained, they "had no comments on our subgroups." He continued:

> We submitted the final statistical analysis plan to the FDA before unblinding the ACT DMD study. However in the RTF

> letter the FDA characterized that PTC proposed a post hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. . . .
>
> We believe the FDA's perspective in the RTF letter may be that although we've pre-specified the subgroup, relying on the subgroup as the main analysis is considered as a post hoc adjustment and we'll be talking to them further on this point.

(AC ¶¶ 98-103)

In August 2016, PTC appealed the RTF, which was denied in November 2016.

D. This Case

From March 3 to March 11, 2017, three class action complaints alleging violations of section 10(b) and Rule 10b-5 were filed against PTC in this district. *See Wang v. PTC Therapeutics, Inc.*, 16-1224, *Parker v. PTC Therapeutics, Inc.*, 16-1384, and *Kosin v. PTC Therapeutics*, 16-1383. Pursuant to the Exchange Act and Private Securities Litigation Reform Act ("PSLRA") of 1995, I consolidated the cases and designated *Wang* as the lead case.

On January 13, 2017, plaintiffs filed a consolidated amended class action complaint ("AC"). Co-lead plaintiff Boston Retirement System is a pension plan which purchased or acquired PTC common stock from November 6, 2014 to February 23, 2016—the "Class Period". A second co-lead plaintiff, Si Nguyen, Hong-Luu Nyugen, John Nguyen, and the Si Tan Nguyen Trust also purchased PTC common stock during the Class Period. So too did another plaintiff, Retail Wholesale Department Store Union Local 338.

Plaintiffs allege that, during the Class Period, PTC knowingly or recklessly made a number of false or misleading statements to investors concerning Translarna. These generally fall into three overlapping categories: (1) statements about the anticipated timeline for FDA review of the 2016 NDA; (2) statements about the likelihood that the ACT DMD trial would meet its

efficacy endpoints; and (3) statements about the ACT DMD results.[9] Under Section 20(a), plaintiffs also seek to hold PTC's CEO, Peltz, and CFO, Kovacs, individually liable as "control persons."

On February 14, 2017, defendants moved to dismiss the AC. The motion is now fully briefed and ripe for decision.

II. DISCUSSION

A. Rule 12(b)(6) and the PLRSA

In place of the normal pleading standard articulated in Fed. R. Civ. P. 8, Plaintiffs pleading securities fraud claims pursuant to Section 10(b) of the Securities Exchange Act and Rule 10b-5 must meet a heightened pleading standard as set forth in the Private Securities Litigation Reform Act ("PLRSA"). 15 U.S.C. § 78u-4(b)(1). Under the PSLRA, plaintiffs bringing a claim involving an allegedly false or misleading statement must: "(1) 'specify each statement alleged to have been misleading [and] the reason or reasons why the statement is misleading,' 15 U.S.C. § 78u–4(b)(1), and (2) 'state with particularity facts giving rise to a strong inference that the defendant acted with the required state of mind,' § 78u–4(b)(2)." *Rahman v. Kid Brands, Inc.*, 736 F.3d 237, 242 (3d Cir. 2013) (quoting *Tellabs, Inc. v. Makor Issues & Rights, Ltd.*, 551 U.S. 308, 321 (2007)) (internal quotations omitted); *accord Williams v. Globus Medical, Inc.*, No. 16-3607, 2017 WL 3611996, at *3 (3d Cir. Aug. 23, 2017). That required state of mind is "scienter," which is defined as "a mental state embracing intent to deceive, manipulate, or defraud." *Rahman,* at 242. (quoting *Tellabs*, 551 U.S. at 319).

Both provisions of the pleading standard require that facts be pleaded "with particularity," echoing the requirement set forth in Fed. R. Civ. P. 9(b). *Institutional Investors Group v. Avaya, Inc.*, 564 F.3d 242, 253 (3d Cir. 2009); see *Fed.* R. Civ. P. 9(b) (a party must state with particularity the

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[9] Both sides have employed this three-part categorization of the allegations, and I do so as well.

"circumstances constituting fraud or mistake."). Although the PSLRA supplanted Rule 9(b) as the pleading standard governing private securities class actions, Rule 9(b)'s particularity requirement is effectively subsumed by the requirements in Section 78u-4(b)(1) of the PSLRA. *Id.* (citing *Miss. Pub. Employees' Ret. Sys. v. Boston Scientific Corp.*, 523 F.3d 75, 85 n. 5 (1st Cir. 2008)). This standard requires that plaintiffs plead the "who, what, when, where and how" of their claims. *Id.* (citing *In re Advanta Corp. Secs. Litig.*, 180 F.3d 525, (3d Cir. 1999)).

Where the PSLRA exceeds the requirements of Rule 9(b), however, is in its approach to pleading scienter.[10] Under the PSLRA, the Court must evaluate whether all the facts in the complaint as alleged, taken collectively, give rise to a "strong inference of scienter." *Tellabs*, 551 U.S. at 323. In determining whether the pleaded facts collectively give rise to a strong inference of scienter, the Court must take into account plausible opposing inferences. *Id.* This involves a comparative inquiry, weighing the likelihood of one conclusion as compared to others, in light of the pleaded facts. *Id.* The Court must therefore consider not just plaintiff-friendly inferences, but also plausible, nonculpable explanations for the defendant's conduct. *Id.* Although the inference that the defendant acted with scienter need not be irrefutable, the inference must be more than merely "reasonable" or "permissible." *Id.* A complaint will survive only if a reasonable person would "deem the inference of scienter cogent and at least as compelling as any opposing inference one could draw from the facts alleged." *Id.*

These PSLRA pleading requirements apply irrespective of whether the alleged fraudulent statement at issue is an assertion of current fact or a prediction of the future. *Avaya*, 564 F.3d at 253-54. When an allegation involves a prediction, however, the Safe Harbor Provision of the PSLRA provides

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[10] Rule 9(b) provides that "[m]alice, intent, knowledge, and other conditions of a person's mind may be alleged generally."

some protection to defendants. The Safe Harbor Provision immunizes from liability any forward-looking statement provided that "the statement is identified as such and accompanied by meaningful cautionary language; or is immaterial; or the plaintiff fails to show the statement was made with actual knowledge of its falsehood." *Id.* at 254; 15 U.S.C. § 78-u-5(c).

B. Analysis

Securities fraud has six elements: "(1) a material misrepresentation or omission, (2) scienter, (3) a connection between the misrepresentation or omission and the purchase or sale of a security, (4) reliance upon the misrepresentation or omission, (5) economic loss, and (6) loss causation." *City of Edinburgh Council v. Pfizer, Inc.*, 754 F.3d 159, 167 (3d Cir. 2014). PTC seeks to dismiss the AC for failure to plead elements (1) and (2).[11] To some extent, PTC is entitled to prevail on its contentions. As to Peltz and Kovacs's allegedly false projections about Translarna's regulatory review timeline and mischaracterizations about the risk that the ACT DMD trial would fail, I agree that plaintiffs have failed to allege factually that the statements were false or misleading when made. But as to Peltz's and Kovacs's allegedly false or misleadingly incomplete statements about what the ACT DMD trial actually demonstrated, and the resulting inability to pass even the most minimal standard of FDA review, the AC contains enough particularized factuality to allege falsity and scienter.

1. Actionable Misrepresentations

As to the first element of securities fraud, PTC argues that the AC fails to allege with sufficient particularity any false or misleading statement. Recall that Peltz's or Kovacs's allegedly false or misleading statements fall into roughly three categories: (1) statements, as of November 2014, about Translarna's review timeline; (2) statements, as of early-to-mid 2015, about risks of the ACT

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[11] PTC does not argue that any of the alleged misrepresentations or omissions are immaterial.

DMD study; and (3) statements, from October 2015 and beyond, about the ACT DMD results. Statements in groups (1) and (2) are not actionable. Group (3) statements are actionable.

i. Timeline Projections

Plaintiffs first point to a couple of statements in which PTC suggested that Translarna would launch by the first half of 2016. Both were made by Peltz during a November 6, 2014 quarterly earnings call:

> 1) "We expect that the submission of this confirmatory Phase 3 data will complete our rolling NDA. . . ."
> 2) "We'll then expeditiously get it in and it's our hope and I think in our dialog with them [*i.e.*, the FDA], given the severe unmet medical needs that this would be rapidly reviewed on that, that this would expect in terms of the approval to move it up potentially after six months. So I think we're thinking about is that we would think this can be a launch within the first half of 2016."

(AC ¶¶ 109-110)

These statements were false or misleading, the AC alleges, because PTC failed to "disclose the substantial risk that the Translarna NDA submission *would be* rejected as facially insufficient by the FDA." (emphasis added). (AC ¶ 111) That the FDA eventually declined to file the 2016 NDA—some 15 months *after* these allegedly false statements were made—does not suggest they were false or misleading as of November 2014. Nor is there any factual allegation that, as of November 2014, the FDA had told PTC something about the design or structure of the ACT DMD study or statistical analysis plan that would have made the facts underlying Peltz's projections false or misleading. Indeed, the ACT DMD study and statistical analysis plan were not even submitted to the FDA until months later, in the spring of 2015. "To be actionable, a statement or omission must have been misleading at the time it was made; liability cannot be imposed on the basis of subsequent events." *In re NACH Inc., Secs. Litig.*, 306 F.3d 1314, 1330 (3d Cir. 2002); *accord Williams*, 2017 WL 3611996, at * 7 ("[I]nstead of citing contemporaneous sources to show

Globus knowingly incorporated Vortex revenue into those projections, plaintiffs rely on conjecture based on subsequent events. This is insufficient.") The AC contains no factual basis to support a conclusion that these statements were false or misleading when made in November 2014.

As to these two statements about Translarna's anticipated FDA review timeline, then, the motion to dismiss is granted.[12]

ii. Risk of the ACT DMD Study

Plaintiffs next point to a handful of alleged misstatements made after the ACT DMD trial had started but before it concluded. Those statements generally implied that there was a low risk that the ACT DMD study would fail meet its endpoints, and a good chance that the study would "confirm" the benefit of Translarna and allow PTC to file successfully an NDA. These statements were false or misleading, plaintiffs say, because PTC knew or learned that the ACT DMD was likely, if not certain, to fail even before it received the final results. Here, too, the AC lacks factual particulars that would make that a plausible allegation.

Here are the four allegedly false or misleading statements.

1) At a January 15, 2015 healthcare conference, Peltz stated:

> "We have the confirmatory trial for Duchenne muscular dystrophy ongoing. That will allow us then to sell it in the United States, where we expect each trial to be completed this year and next year that we get approval in the United States . . . What we did is we used the learnings from the previous studies to really wring out the risk in the current study."

(AC ¶¶ 113-14)

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12 Because the AC does not adequately allege that Peltz's statements about Translarna's regulatory timeline were false when made, I do not reach PTC's argument that they were protected by the PSLRA's Safe Harbor Provision, which governs forward-looking statements.

2) At a March 9, 2015 healthcare conference, PTC senior vice president Tuyen Ong stated:

> I think we talked about the confirmatory studies really being enriched and sort of enhanced and somewhat we've wrung out the risk of the confirmatory study based on the learnings of the second study. I think ultimately it's really dependent on the data; is there a clinical benefit, is it statistically significant.

(*Id.* ¶ 118)

3) At a May 6, 2015 healthcare conference, Kovacs stated:

> [O]bviously we've tried to mitigate as much risk as possible in this study by a lot of the care around reducing the enrollment criteria and try to control for the patients that were at least enrolled in the study . . . So we're doing that (inaudible) de-risk the outcome.

(*Id.* ¶ 121)

4) At a May 12, 2015 healthcare conference, Peltz stated:

> [I]f you think about what have we done and refined for this study versus the prior Phase 2 study that gives us such a high degree of confidence in the likelihood of a positive outcome in this study later this year . . . .

(*Id.* ¶124)

Plaintiffs again allege that these four statements were false and misleading because PTC "failed to disclose the substantial risk that the Translarna NDA submission *would be* rejected as facially insufficient by the FDA." (*Id.* ¶¶ 116, 119, 122, 125) (emphasis added)). Describing the ACT DMD study as "confirmatory" was likewise misleading, the AC alleges, because the FDA "*would* require PTC to demonstrate Translarna's efficacy more sufficiently than the company had in its Phase 2b trials." (*Id.*) (emphasis added). What PTC ultimately failed to disclose, plaintiffs say, is that "the design of the ACT DMD study had just as much risk of failure as had the Phase 2b trial," and did not "minimize[] any risk of negative outcomes." (*Id.* ¶¶ 117, 120, 123, 125)

While it is true that the FDA eventually rejected the 2016 NDA based on the facial insufficiency of the ACT DMD results, the AC alleges no facts to suggest that PTC knew that would happen *as of January, March and May 2015.* Reasoning backward from the eventual rejection, plaintiffs posit that the ACT DMD must have been doomed from the start. To plead an actionable statement, however, a securities fraud complaint must contain particularized facts that plausibly suggest that a misstatement was false or misleading when made; it is never enough to plead "fraud by hindsight." *NAHC*, 306 F.3d at 1330; *Cal. Pub. Employees Re. Sys. v. Chubb Corp.*, 394 F.3d 126, 158 (3d Cir. 2004) ("We have long rejected attempts to plead fraud by hindsight."); *cf. Tellabs*, 551 U.S. at 320 ("The 'strong inference' formulation was appropriate, the Second Circuit said, to ward off allegations of 'fraud by hindsight.'"). The AC here contains no such facts, however.

Plaintiffs proffer that the FDA had told PTC that the trial would need to meet certain "additional" requirements to pass muster, and that PTC knew it couldn't despite its enriched patient population and pre-specified subgroups. Plaintiffs never specify the content of those requirements, or state who knew about them and when. Their very existence, and PTC's knowledge of them, is simply assumed. More is required to bridge the gap between conceivability and plausibility.

The particularity requirement of the PSLRA and Rule 9(b) is more stringent than the usual pleading standard, if not actually onerous: What is required is the level of factual detail ordinarily found "in the first paragraph of any newspaper story." *Avaya*, 564 F.3d at 253. As to PTC's statements about the ACT DMD study's risk, the AC fails to make those minimal factual allegations: what the FDA told PTC, when it did so, and how that that information related to the likelihood of the study's success. The AC therefore

fails to allege plausibly that Peltz and Ong's statements about the risk of the ACT DMD study were false or misleading when made.[13]

The motion to dismiss as to this second group of statements is therefore granted.[14]

iii. ACT DMD Results

The third and final group of allegedly false or misleading statements stands on a different footing. These statements concern the actual results of its clinical trials. The AC alleges that although PTC knew that the ACT DMD trial—like the 2b trial—had failed to meet its primary endpoints for most patients, PTC stated or implied that the "totality" and "consistency" of the clinical data met the requirements for FDA approval. Unlike PTC's statements about Translarna's review timeline or the likelihood of the ACT DMD study's success, these were not predictions. The AC plausibly alleges that PTC's statements about the ACT DMD results were factual in nature, and were misleading or false when made.

Here are the specific statements.

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13 In this way, this case is very different from *In re Nuvelo, Inc. Secs. Litig.*, 668 F. Supp. 2d 1217 (N.D. Cal. 2009). The complaint in that case alleged that the defendant had agreed with the FDA "that regulatory approval rested on achieving . . . a p-value of 0.00125," but told investors that it had modelled its study after a program that used a 0.05 p-value. That type of concrete factual averment, which plausibly suggests falsity, is absent from the AC.

The facts the AC *does* contain actually suggest that some of PTC's statements about the risk of the ACT DMD were not misleading at all. By pre-specifying two subgroups it thought might especially benefit from Translarna, PTC *did* "use the learnings of the previous studies" to "mitigate" the risk of the ACT DMD study. By restricting enrollment in the Act DMD study to patients of certain age who could walk a certain distance, PTC *did* "enhance[]" and "enrich[]" the pool of patients in the ACT DMD study. Knowing what we know now, it was overly optimistic for Peltz to proclaim that PTC had "really wr[u]ng out the risk" of the ACT DMD study. Nevertheless, as Ong told investors in March 2015, whether the ACT DMD study would provide substantial evidence of Translarna's effectiveness was "really dependent on the data; is there a clinical benefit, is it statistically significant."

14 Here, too, I need not address PTC's alternative argument that forward-looking statements about the risk of the ACT DMD study qualify for safe harbor protection.

1) In an October 15, 2015 conference call announcing the ACT DMD results, Peltz told analysts and investors:

> [W]e are very pleased that the totality of the Translarna results demonstrate clinical benefit for DMD. These include ITT results, the pre-specified subgroup results, and pre-specified meta-analysis . . . .
>
> * * *
>
> The totality of the data for Translarna demonstrates clinical benefit across primary and secondary endpoints. We have pre-specified the key subgroup for analysis and meta-analysis, both of which show Translarna had a clinically meaningful benefit for DMD patients. The results of the ACT DMD trial show consistent evidence of the clinical benefit of Translarna for individuals with nonsense mutation Duchenne muscular dystrophy . . . .
>
> * * *
>
> The pre-specified meta-analysis was in our statistical analysis plan, which we had discussions with with [*sic*] the FDA. This was in part, part of the pre-specified plan. So they are well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure. . . .
>
> * * *
>
> We are proud to have confirmed the benefit of Translarna for the DMD patients.

(AC ¶¶ 126-130)

2) A November 9, 2015 press release stated:

> ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy. . . . The totality of the clinical data from two large, placebo-controlled clinical trials across over 400 patients demonstrates Translarna's ability to slow disease progression.

(*Id.* ¶ 136)

3) A quarterly earnings report filed the same day similarly said:

> [W]e believe that the results of ACT DMD and the totality of clinical data across our two large, randomized, placebo-controlled trials (ACT DMD and our prior Phase 2b study, Study 007), provide substantial evidence of the effectiveness of Translarna and demonstrate a clinically meaningful benefit of Translarna for the treatment of nmDMD.

(AC ¶ 137)

4) Peltz, during conference call announcing the quarterly earnings report, told participants:

> The goal is to show efficacy with given endpoints . . . . We see this in ACT DMD. . . . [T]he totality of clinical data confirmed Translarna's ability to slow disease progression for patients with DMD.

(*Id.* ¶ 138)

5) At a November 18, 2015 healthcare conference, Kovacs said:

> And the big picture about our data is and what will be part of our argument to both the regulatory authorities in the US and Europe is that the consistency of the results now seen across two of the largest placebo-controlled Phase 3 studies ever done in the disease, the totality of the data support the clinical benefit and certainly the risk-benefit profile of the drug in favor of an approval and getting something to these kids.

(*Id.* ¶ 143)

6) At a December 19, 2015 healthcare conference, Kovacs stated:

> Our intention today is for filing for full approval on the basis of two large well-controlled studies that all point to safety and efficacy for a risk-benefit profile in favor of the drug.

(*Id.* ¶ 147)

7) On January 13, 2016, Peltz told attendees at a healthcare conference:

> So you can see in two large studies where we used the six minute walk test as the primary endpoint, we saw a benefit both in the primary endpoint as well as secondary endpoints. And in prespecified subgroups, we saw more robust effects being observed, both the primary and secondary endpoints. So consistent data in two independent studies.
>
> One of the things we've noticed they asked for was a sensitivity analysis, and that while you have prespecified subgroups, if you go beyond those, does the data still show clinically meaningful differences? And it does both in the primary and secondary endpoints.

(AC ¶ 150)

8) At the same conference, Peltz said of one of the meta-analyses:

> In the meta-analysis, where you combine the results, you see both in the six-minute walk distance as well as the time function tests, you see clinically meaningful and statistically significant improvements with Translarna over placebo. . . .
>
> And really it's consistent with the totality of the data, demonstrating that this drug was efficacious. So, I think we've checked that box.

(*Id.* ¶ 151)

All eight statements generally claim that the "totality" or "consistency" of the data "show Translarna had a clinically meaningful benefit for DMD patients," "demonstrates Translarna's ability to slow disease progression," "provides substantial evidence of the effectiveness of Translarna," and "point to safety and efficacy for a risk-benefit profile in favor of the drug." Yet, as the AC alleges, *only a fraction of patients*—about 25%—ever reported a clinically meaningful and statistically significant benefit in the 6-minute walk test across the ACT DMD and 2b studies on a pre-specified basis. Nor did the pre-specified

meta-analysis, which reported a statistically significant but not clinically meaningful benefit, demonstrate "consistent" clinical benefit across the ACT DMD ITT and decline phase 2b populations. Contrary to PTC's representations, then, the "totality" of the data *did not* "demonstrate clinical benefit for DMD" or evince "consistent" evidence of "clinical benefit" since each study and the pre-specified meta-analysis *failed to produce such evidence for most DMD patients.*[15]

If defendants' assertions about the clinical data were were true, the AC alleges, it was because of the post-hoc meta-analysis of the 300-400 meter subgroup. Yet that meta-analysis *was not part of the pre-specified ACT DMD statistics plan.* As PTC had learned five years earlier, however, post-hoc adjustments to clinical data are no substitute for a well-controlled clinical trial meeting its primary efficacy and statistical significance endpoints.[16] More importantly, plaintiffs allege, PTC never disclosed that the 300-400 meter subgroup meta-analysis was the primary factual support for its public statements.[17]

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[15] I note that statement (7), that data *outside* of the pre-specified subgroups demonstrated "clinically meaningful differences," will bear an interpretation that is affirmatively false, since the ACT DMD study *failed* to meet its endpoints for the ITT population. (The statement might also be misleading, if the <350 meter subgroup failed to reach its endpoints too). Similarly problematic is the part of statement (1) in which Peltz claimed that the FDA had "agreed" upon the ACT DMD pre-specified meta-analysis, if the point of that statement was to suggest that the FDA would find that meta-analysis sufficient to move past facial review. Another arguably misleading part of statement (1) is the assertion that PTC "pre-specified the key subgroup for analysis, both of which show Translarna had a clinically meaningful benefit . . . ." That implies that there was only one pre-specified subgroup when there were two, and that the pre-specified meta-analysis revealed a clinically meaningful benefit, which it didn't.

[16] I do not suggest that the use of a meta-analysis is inherently false or misleading. The technique has its place. The thrust of the FDA's position would seem to be that it carries the danger of substituting wishful thinking for scientific rigor; the inevitable cliché is that it is comparable to shooting an arrow at a wall and then drawing a target around it. Plaintiff's real point, however, seems to revolve around the allegedly inadequate disclosure of the extent to which PTC was relying on a post hoc meta-analysis of the data.

[17] As early as Peltz's October 15, 2015 remarks, PTC used the 300-400 meter post-hoc analysis as evidence that the ACT DMD study "confirmed" the benefit of Translarna and "demonstrated consistent benefit," even though Peltz allegedly implied

PTC says that these statements were not misleading because, at least for some of the statements, it disclosed that the ACT DMD study failed to meet its endpoints for the overall ITT population.[18] *See, e.g., Kleinman v. Elan Corp., plc*, 706 F.3d 145, 153 (2d Cir. 2013) (finding no actionable misstatement where defendant emphasized "encouraging preliminary findings" found in a subgroup but "disclosed that the 'overall study population' did not attain statistically significant results based on primary endpoints'"). But PTC was not merely highlighting favorable data while downplaying disappointing data—it was affirmatively telling investors that it had proven that Translarna was effective at treating DMD. Arguably, the actual results of the 2b and ACT DMD studies were actually contrary, which suggests a plausible allegation that PTC made "affirmative false statements about a drug's efficacy and safety" to lull investors into thinking that the clinical data *was* sufficient to meet FDA efficacy standards. *City of Edinburgh Council v. Pfizer*, 754 F.3d 159, 170 (3d Cir. 2014) (citing *In re Viropharma Inc., Litig.*, No. CIV.A. 02-1627, 2003 WL 1824914, at *4, 7 (E.D. Pa. April 7, 2003) ("Statements regarding the overall efficacy of the drug . . . cannot be simply dismissed as immaterial . . . . [T]he Plaintiffs have pleaded that statements by Defendants were contrary to the then existing state of facts, for example, that Pleconaril was effective for all adults when it was not.")).[19]

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that the sole factual bases for his statements were the ITT, the subgroup, and pre-specified meta-analysis results. (*Compare* Def. Ex. 23 p. 3 *with* Def. Ex. 24) Defendants, to be sure, disclosed in its November 9, 2015 10-Q that its "our conclusions regarding the . . . potential efficacy of Translarna in nmDMD are primarily based on pre-specified meta-analysis and ACT DMD data and retrospective analyses of our Phase 2b clinical data . . .." (Def. Ex. 15, pp. 44) Again, however, this disclosure fails to inform investors that PTC was relying on the post-hoc meta-analysis as the "main analysis."

[18] *See, e.g.* Def. Exs. 13, 15, 23. A number of the allegedly false statements were made orally at healthcare conferences, however, and PTC has not submitted transcripts of those remarks.

[19] PTC cites *Pfizer* for the proposition that "interpretations of clinical data are considered opinions" and "[o]pinions are only actionable under the securities laws if they are not honestly believed and lack reasonable basis." 754 F.3d at 170. Only one

Furthermore, PTC did not disclose that the primary (and perhaps only) factual support for its claim that "totality" and "consistency" of the ACT DMD and 2b data was the post hoc meta-analysis of the 300-400 meter subgroup.[20] Had PTC made such a disclosure, plaintiffs allege, the factual parallels between PTC's failed 2011 NDA effort and its after-the-fact manipulation of the ACT DMD would have been obvious to a reasonable investor.

PTC points out that Section 10(b) and Rule 10b-5 "do not create an affirmative duty to disclose any and all material information." *Pfizer*, 754 F.3d at 173-74. That is true, as far as it goes. *Id.* at 174 ("A duty to disclose under federal securities laws may arise when a statute requires disclosure, insider trading occurs, or there is an inaccurate, incomplete, or misleading prior disclosure.") (citing *Oran v. Stafford*, 226 F. 275, 285-86 (3d Cir. 2000). But "once a company has chosen to speak on an issue—even an issue it had no independent obligation to address—it cannot omit material facts related to that issue so as to make the disclosure misleading." *Williams*, 2017 WL 3611996, at *4 (quoting *Kline v. First W. Gov't Sec., Inc.*, 24 F.3d 480, 490-91 (3d Cir. 1994)

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of the alleged misstatements (contained in a document drafted by a lawyer) is potentially a statement of opinion, however. (AC ¶ 137 ("[W]e *believe* that the results of ACT DMD and the totality of clinical data . . . provide substantial evidence of the effectiveness of Translarna . . . .") (emphasis added)). And even that statement allegedly omitted that the factual basis for that statement was a non-pre-specified meta-analysis of the 300-400 meter subgroup. More fundamentally, these statements were not misleading because they were opinions that were not honestly believed and lacked a reasonable basis (though they may well have been). Plaintiffs instead allege that they were false or misleading because they are misrepresented the existing state of facts (*i.e.*, whether the clinical *actually* provided substantial evidence of effectiveness, whether subgroups outside the 300-400 meter subgroup *actually* reported a clinically meaningful benefit, etc.).

[20] Contrary to its assertions in the papers, PTC did not "accurately disclose that the only positive results from the entirety of the . . . study stemmed from the use of post-hoc analysis." *Kleinman*, 706 F.3d at 154-55. Rather, it implied, or affirmatively stated, that the "totality" and "consistency" of *the pre-specified ACT DMD results* provided "substantial evidence" of effectiveness. From Peltz's October 15, 2016 remarks especially, it was not at all clear "that a post-hoc analysis [was] being used" to support PTC's assertions, let alone that PTC would use such an analysis *as the main analysis* to convince the FDA of Translarna's efficacy. *Id.* at 154-55.

("[E]ncompassed within that general obligation [to speak truthfully] is also an obligation to communicate any additional or qualifying information, then known, the absence of which would render misleading that which was communicated.") Such is the case here. According to the AC, PTC omitted facts, including the extent to which it was relying on post-hoc statistical analysis, which made PTC's disclosures misleading. Those allegations, taken as true, are sufficient to trigger a duty to disclose.

Because the alleged misstatements are assertions of current fact, not predictions, they do not qualify for safe harbor protection.[21] *In re Viropharma Inc. Sec. Litig.*, 21 F. Supp. 3d 458, 471 (E.D. Pa. 2014) ("[O]missions of existing facts or circumstances are not forward-looking, and thus do not qualify for safe harbor protection."); *see also* 15. U.S.C. § 78u-5(i)(1)(A)-(C) (defining forward-looking statements to include "a projection of revenues, income (including income loss), earnings (including earnings loss) per share, capital expenditures, dividends, capital structure, or other financial items"; statements of "the plans and objectives of management for future operations, including plans or

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[21] Even assuming they were predictive, they still would not qualify for safe harbor protection, at least not on the basis of what is alleged in the AC. Statements (1), (4), (5), (6), (7), and (8) were made orally, and the parties have not performed the necessary analysis to allow me to rule on this issue at this time. *See EP Medsystems, Inc., v. EchoCath, Inc.*, 235 F.3d 865, 872-73 n.3 (3d Cir. 2000) (oral forward-forward looking statements may qualify for safe harbor protection if the speaker tells the audience that the statement is forward-looking if "(1) the issuer informs the audience that the statement is forward-looking and that actual results may differ materially from the predictions; (2) the issuer orally directs the audience to other "readily available" written documents that contain the additional information about important factors relating to the forward-looking statement; and (3) the identified documents set forth satisfactory cautionary statements.") (citing 15 U.S.C. § 78u-5(c)(2)(B)).

While statements (2) and (3) are written, they are not accompanied by meaningful disclosures. The relevant disclosures are boilerplate recitations of the possibility that the FDA may not "agree with our interpretation of the results of ACT DMD" and that retrospective analyses "are generally considered less reliable than pre-specified analyses." (Def. Ex. 14, p. 4, Def. Ex. 15 p. 44) That language, however, is not "extensive, specific, and directly related to the alleged misrepresentations," *In re Aetna, Inc. Sec. Litig.* 617 F.3d 272, 278-79 (3d Cir. 2010), which in this case includes PTC's allegedly exclusive reliance on 300-400 meter post-hoc meta-analysis.

objectives relating to the products or services of the issuer"; or statements of "future economic performance").

In sum, taking the factual allegations of the AC as true and drawing all inferences in plaintiffs' favor, I find that there are sufficient factual particulars to support the allegation that PTC misrepresented the sufficiency of the ACT DMD and 2b results to meet even the most basic FDA review standards. As to these statements, then, the motion to dismiss for failure to plead an actionable misstatement is denied.

* * *

To summarize, plaintiffs have not adequately alleged the reasons why PTC's late 2014 and early 2015 statements about (1) Translarna's FDA review timeline and (2) the risk of the ACT DMD study were false or misleading when made. I find sufficient the allegations that (3) PTC misrepresented the ACT DMD results. The motion to dismiss is therefore granted as to statements (1) and (2) but denied as to (3).

2. Scienter

I next consider the issue of whether the AC adequately alleges that Peltz's and Kovacs's statements concerning the ACT DMD results and the "totality" and "consistency" of the clinical data were made with scienter.

Under the PSLRA, it is not enough to plead that a defendant has made a misleading or false statement. The complaint must also plead that the alleged misrepresentations were made with the "intent to deceive, manipulate, or defraud." *Rahman*, 736 F.3d at 242. "This scienter standard requires plaintiffs to allege facts giving rise to a 'strong inference' of 'either reckless or conscious behavior." *Avaya*, 564 F. 3d at 267-68 (footnote omitted). Recklessness in this context is an "extreme departure from the standards of ordinary care, and which presents a danger of misleading buyers or sellers that is either known to the defendant or is so obvious that the actor must have been aware of it." *Id.* at 267 n. 42 (quoting *Advanta* 180 F.3d at 535). While the factual particulars pled

must give rise to "a strong inference" of scienter, the inference "need not be irrefutable, *i.e.*, of the 'smoking gun' genre, or even the most plausible of competing inferences." *Id.* at 267 (quoting *Tellabs*, 551 U.S. at 326 (2007)). "The pertinent question is whether *all* of the facts alleged, taken collectively, give rise to a strong inference of scienter, not whether any individual allegation, scrutinized in isolation, meets that standard." *Id.* (quoting *Tellabs*, at 323.)

Viewed holistically, the AC alleges sufficient circumstantial evidence that PTC's alleged misstatements about the ACT DMD results were made with the necessary fraudulent intent. I again emphasize, of course, that these are allegations only, untested by discovery or the adversary process.

Most persuasive here are the content and context of the alleged misstatements. *See Avaya*, 564 F. 3d at 269 ("Among the facts alleged by Shareholders, the most powerful evidence of scienter is the content and context of [defendant's CFO's] statements themselves."). During conference calls, public remarks, and in quarterly reports, Peltz and Kovacs explicitly and repeatedly told investors that the "totality" and "consistency" of the ACT DMD and 2b data "demonstrates Translarna's ability to slow disease progression" and provides "substantial evidence" of effectiveness. (*E.g.* AC ¶¶ 126, 128, 136, 138, 143) At the time those statements were made, however, Peltz and Kovacs knew that the FDA refused to file the 2011 NDA because the "2b clinical trial . . . did not achieve statistical significance in the pre-specified analysis." (*Id.* ¶ 65) They knew that the ACT DMD trial, which was stacked with what were considered ideal patients, not only failed to reach its primary endpoints for the ITT population, but generally reported *worse* results than the 2b study. (*Id.* ¶¶ 72, 81) They knew that the FDA typically requires two successful clinical trials before approving a drug, and that Translarna had none. (*Id.* ¶ 49) And they knew that the 300-400 meter post hoc meta-analysis, which was the most persuasive evidence of clinically meaningful and statistically significant effect across all trials, was the same type of non-pre-specified statistical analysis that the FDA had previously found facially insufficient. (*Id.* ¶¶ 52, 62-63, 65, 85, 88,

89, 100) These facts strongly suggest that Peltz and Kovacs knew or recklessly disregarded the obvious risk that their statements would mislead the investing public as to whether the ACT DMD results met FDA efficacy standards.[22]

PTC, it is true, disclosed that the ACT DMD and 2b trials failed to reach their endpoints and disclosed that the FDA considered post-hoc statistical analyses less reliable than pre-specified ones. And investors knew, as early as 2012, that "the adequacy of the data for filing and approval of an NDA would remain review issues."[23] (AC ¶ 169) Yet the essential allegation here is that PTC knew (or recklessly disregarded the obvious risk) that neither the pre-specified data nor the post hoc data, considered alone or collectively, were even facially sufficient. Nevertheless, the AC alleges, Peltz and Kovacs repeated and confidently told investors that the "totality" and "consistency" of the clinical data met FDA standards. Those statements are hard to square with the facts allegedly known to PTC, including that the ACT DMD had failed on its own terms, and that the FDA would not accept a post hoc statistical analysis as a substitute for a successful clinical trial. These facts, if true, suggest more than

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22 Because these allegations are not supported by testimony from confidential witnesses or documents, PTC seems to suggest that dismissal is appropriate on that basis. It cites no authority holding that such documents are required to state a claim, however. That is not surprising: The documents most likely to prove scienter are commonly within the exclusive control of the defendant, and hence unavailable to plaintiffs at the pleading stage. When the AC was filed, for example, PTC had yet to release the full ACT DMD dataset.

23 During the October 15, 2015 conference call announcing the ACT DMD results, PTC points out that Peltz told investors that their "approach [in pre-specifying the <350 meter and 300-400 meter subgroups] was consistent with the recent FDA draft guidance for Duchenne muscular dystrophy . . . ." (Def. Ex. 23 p. 4). The implication seems to be that PTC had some reason to believe that the FDA might file the 2016 NDA solely on the basis of pre-specified subgroup data if the ACT DMD narrowly missed meeting its ITT endpoints. That may be, though I note that the ACT DMD did not narrowly miss its primary endpoints, it completely missed them. Defendants at any rate will have the opportunity to explore these issues during discovery. As things stand now, however, I do not have a copy of that FDA "draft guidance," and even if I did, the AC alleges that the "main analysis" of the 2016 NDA was post-hoc meta-analysis, not pre-specified subgroup data. The citation to FDA guidance, then, does not quite explain away plaintiffs' allegations.

mere optimism, and support a strong inference that Peltz's and Kovacs's statements were made knowingly or recklessly.[24]

The plaintiffs plead other circumstantial evidence of scienter as well. RTFs, the AC alleges, are relatively uncommon.[25] They are intended to address "omissions or inadequacies so severe as to render the application incomplete on its face." (AC ¶ 52) Because an RTF is based on an NDA's facial sufficiency, not interpretations or judgment calls about what the data says, communicating with the FDA prior to submitting an NDA may diminish the risk of receiving an RTF.[26] (AC ¶54) PTC certainly understood the importance of FDA feedback; it made sure investors knew, for example, that the FDA had blessed ACT DMD study's design and statistical analysis plan. (AC ¶¶ 69, 168) Yet PTC chose *not* to meet with the FDA after receiving the ACT DMD results but before submitting Translarna's 2016 NDA. If PTC had done so, the AC alleges, the FDA would have told PTC that the ACT DMD data was facially inadequate, and that it should not (or should not yet) file the NDA. PTC's silence could support an inference that PTC thought that more discussion and disclosure would only

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24 That the market took Peltz's and Kovacs's representations about the ACT DMD data seriously is corroborated by the precipitous drop in PTC's stock price after, but not before, the 2016 RTF was received.

25 Citing to *In re Sanofi Sec. Litig.*, 87 F. Supp. 3d 510 (S.D.N.Y. 2015) and *In re Bristol Meyers Squibb Secs. Litig.*, 312 F. Supp. 2d 549 (S.D.N.Y. 2004), PTC claims that an RTF in and of itself is not a basis to infer fraudulent intent, and I agree. An RTF, after all, might issue for relatively mundane reasons, such as failing to submit "a completed application form" or presenting data in a confusing way. *See Sanofi*, 87 F. Supp. 3d. at 522. That is not the case here, however. The AC alleges that the FDA issued the 2016 RTF because the clinical data was an insufficient substitute for a successful clinical trial—the same reason the FDA issued the 2011 RTF. Neither *Sanofi* nor *Bristol Meyers* address that kind of allegation. More fundamentally, the RTFs are not the sole basis for the fraudulent intent inference; there is, for example, the content and context of the alleged misstatements themselves.

26 That is especially true for drugs in the fast track program, the AC alleges, which is premised on "early" and "frequent" communication with the FDA. (AC ¶ 13)

harm what was a weak application, but the inference is far from ironclad. I therefore assign it only minor significance.[27]

I also consider certain characteristics of the PTC and the speakers of the alleged misstatements, PTC's founder and CEO, Peltz, and CFO, Kovacs. *See Nat'l Junior Baseball League v. Pharmanet Dev. Grp. Inc.*, 720 F. Supp. 2d 517, 556 (D.N.J 2010) ("[A] person's status as a corporate officer, when considered alongside other allegations, can help support and inference that this person is familiar with company's most important operations."); *see also Avaya*, 564 F.3d at 569-71. Financially, Translarna accounted for 100% of PTC's revenues in February 2016. Short term, Translarna was the only drug PTC could market. Long-term, if the FDA approved Translarna, PTC thought that it could apply its post-transcriptional research to treat other nonsense mutation disorders (and therefore market and sell new drugs). Translarna, in short, was everything to PTC. (AC¶¶ 184, 189)

Inadvertence or inattention, then, do not suggest themselves as alternatives to scienter. It seems implausible that Peltz and Kovacs were not paying close attention to the results of the company's most critical clinical trial for their most important drug. Nor is it plausible that Peltz and Kovacs, as CEO/founder and CFO of PTC, played a subordinate role in the decision to submit the 2016 NDA. To the contrary, Kovacs's and Peltz's statements to investors during earnings calls and healthcare conferences implied that they had first-hand knowledge of ACT DMD results and PTC's conversations with the FDA.[28] These circumstances, while not conclusive, tend further to bolster

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[27] I similarly give minor weight to the allegation that the 2016 NDA sought approval for all DMD patients, as opposed to a specific subgroup of patients.

[28] For that reason, PTC's assertion that the "core operations" doctrine does not support scienter unless "allegations of specific information conveyed to management and related to fraud" are alleged fails. *Rahman*, 736 F.3d at 246. Through their public statements, Peltz and Kovacs demonstrated personal knowledge of the ACT DMD results and PTC's conversations with the FDA.

This case is indeed factually closer to *Avaya*, in which the Third Circuit recognized the core operations doctrine where a company's CFO consistently and

the inference that either Peltz or Kovacs "knew at the time that his statements were false or was reckless in disregarding the obvious risk of misleading the public." *Avaya*, at 273.[29]

PTC resists this conclusion by characterizing these allegations as a species of "fraud-by-hindsight." I disagree. The short answer, of course, is that the AC alleges that Peltz and Kovacs knew, but repeatedly underplayed, a number of then-existing facts that all suggested that the risk of FDA disapproval was much greater than they let on. That is an allegation of classic

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repeatedly denied the existence of price competition during conference calls with analysts when the company was actually engaged in "widespread discounting involving many different product lines and accounts, including some of Avaya's largest clients." *Avaya*, 736 F. 3d at 269-70. "Because of the context (specific analyst inquiries) and content (consistent denials of unusual discounting) of [the CFO's] statements, the possibility that McGuire was ignorant is not necessarily exculpatory." *Id.* at 270. Here, as there, even if Peltz or Kovacs "were not aware of the full extent" of the risk that the FDA would find the clinical data facially insufficient, they "might be culpable as long as what [they] knew made obvious the risk that [their] confident, unhedged" assertions would mislead investors. Taking the factual allegations of the AC true, what Kovacs and Peltz knew about the ACT DMD trial—that most patients *did not* report a statistically significant clinical benefit from Translarna—suggests a strong inference that their statements that "totality" and "consistency" of the clinical data demonstrated substantial evidence of efficacy reached this required level of recklessness.

[29] I give less weight to the AC's allegations of motive and opportunity. The AC alleges that Peltz and Kovacs knew that devising and implementing another clinical study would be costly and expensive, and could jeopardize PTC's financial success. "[M]otives that are generally possessed by most corporate officers do not suffice," however. *Rahman*, 736 F.3d at 245-46 n. 13 (citing *Avaya*, 564 F.3d at 278-79 ("Corporate officers always have an incentive to improve the lot of their companies, but this is not, absent unusual circumstances, a motive to commit fraud."). The "absence of a motive allegation is not fatal" to a securities fraud claim, so long as the complaint adequately alleges a strong inference of scienter, which this complaint does. *Tellabs*, 551 U.S. at 325.

Because I find that Peltz and Kovacs made the alleged misstatements with scienter, I do not reach defendants' closely related argument that plaintiffs fail to plead "corporate scienter." *E.g.*, *Rahman*, 736 F.3d at 246 (citing *Teamsters Local 445 Freight Div. Pension Fund v. Dynex Capital Inc.*, 531 F.3d 190, 195 (2d Cir.2008) ("In most cases, the most straightforward way to raise such an inference for a corporate defendant will be to plead it for an individual defendant."). This is allegedly a case of direct statements by a corporate CEO and CFO, not one of diffuse organizational responsibility.

fraud, not fraud-by-hindsight. The somewhat longer answer is that cases in which district courts in this circuit have dismissed "fraud-by-hindsight" claims against pharmaceutical companies based on what those companies "must have known" about clinical data and approval prospects are not quite on point.

PTC cites four cases in which allegations that defendants knew but misrepresented certain information about clinical data, which ultimately misled investors about the likelihood of FDA approval, were insufficient to repel a motion to dismiss. *See In re Columbia Laboratories, Inc. Secs. Litig.*, Civ. No. 12-614, 2013 WL 5719599 (D.N.J. Oct. 21, 2013); *Sapir v. Averback, et al.*, Civ. No. 14-7331, 2016 WL 554581 (D.N.J. Feb. 10, 2016); *In re Adolor Corp. Secs. Litig.*, 616 F. Supp. 2d 551 (E.D. Pa. 2009); *In re Amarin Corp. PLC*, Civ. No. 13-6663, 2015 WL 3954190 (D.N.J. June 29, 2015).[30] Those cases, however, lack key factual particulars alleged in this case. For example, none of the drugs at issue in *Columbia, Sapir, Adolor,* or *Amarin* ever received an RTF, let alone two RTFs issued for essentially the same reason. More importantly, the *Columbia, Sapir, Adolor,* and *Amarin* complaints rested on allegations as to what the defendants "must have known" about their clinical data; the AC here, by comparison, contains specific factual allegations about what Peltz and Kovacs *actually* knew at the time the alleged misstatements were made.

PTC alternatively argues that an inference of non-fraudulent intent is stronger than any inference of fraudulent intent. Essentially, PTC contends

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30 *See Columbia*, 2013 WL 5719500, at *7 (rejecting allegations that defendants knew that a clinical trial did not demonstrate efficacy because there was no factual allegation that the FDA had told defendants that the results would need to meet a certain p-value threshold); *Sapir*, 2016 WL 554581 at *10-14 (rejecting allegations that defendants knew that their Phase 3 study would fail because documents flatly contradicted plaintiff's proposed inference); *Adolor*, 616 F. Supp. 2d at 562-63, 575-76 (rejecting allegations that defendants knew that it had "rigged" and "manipulated" clinical data because "Defendants' public disclosures accurately described the results of each study"); *Amarin*, 2015 WL 3954190, at *5-8, 13-15 (rejecting allegations that defendants were aware of but chose to ignore concerns with clinical trials because they "merely create[d] the inference . . . [that] executives were optimistic about the success of Vascepa . . ., despite some concerns").

that it is not plausible that it would file an NDA it knew would fail. The facts, it says, were not so one-sided. The pre-specified 300-400 meter subgroup—a sizable group of about 100 patients—reported statistically significant and clinically meaningful results, while the pre-specified meta-analysis demonstrated a modest statistically significant (if not clinically meaningful) benefit. Because of the significant unmet need for a DMD treatment, PTC says, it was reasonable to think that FDA might approve Translarna regardless of the ACT DMD results if the potential benefits outweighed safety concerns. And not for nothing, PTC points out that it had been selling Translarna in Europe since 2014 solely on the basis of the 2b trial data.[31] Given these facts, defendants say, the more compelling inference is that Peltz and Kovacs believed what they said, even though they were ultimately wrong about what the FDA would require.[32]

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31 Defendants also point to two competitor's NDAs, which the FDA filed despite those companies' failures to either complete a confirmatory trial or meet the primary endpoints of such a trial. Because, as noted above, the relevance of these NDAs to the issues presented here rest on a host of factual assumptions and inferences, I give these facts little weight at this early pleading stage.

32 With some force, defendants claim that this case is the mirror image of *Kuyat v. BioMimetic Therapeutics*, 747 F.3d 435 (6th Cir. 2014). The plaintiffs there alleged that BioMimetic misrepresented that ITT results were less important than modified ITT results, even though the "FDA privately communicated" to the company that it "expected BioMimetic to obtain statistically significant results based on an analysis of the ITT population." *Id.* at 438-41.

Affirming the trial court's dismissal of the complaint, the Court of Appeals for the Sixth Circuit observed that there were a number of reasons why "BioMimetic rightfully expressed optimism about the device's success," including the FDA's prior approval of other devices based on mITT analyses and the device's approval had been in Canada and Australia. *Id.* at 442-43. The complaint also failed to show that the "presence or absence of statistically significant results in an analysis of the ITT population was the FDA's absolute requirement" generally or the FDA's position as to Augment specifically. *Id.*

This AC is different. The AC factually alleges that the FDA typically requires two successful clinical trials before approving a drug, which Translarna didn't have, and that PTC knew from prior experience that post-hoc meta-analyses would not substitute for pre-specified clinical data. Now it is true, as defendants point out, that the AC lacks factual particulars about what the FDA may have "privately" communicated to PTC. But the AC alleges that the FDA refused to file Translarna's

There is something to be said for the defendants' account, and PTC will have an opportunity to adduce evidence in support of it at a later stage. For now, however, it is sufficient to note that PTC's version of events fails to negate key facts, including its consistent and confident assertions about what the clinical data actually said (as opposed to what it believed or interpreted the data said) and its sufficiency to meet FDA requirements. Nor does PTC's proposed inference negate its failure to explain that the post hoc 300-400 meter subgroup meta-analysis was the "main analysis" supporting Peltz's and Kovacs's assertions that the "totality" and "consistency" of the evidence evinced substantial evidence of effectiveness. That is not to say that plaintiff's version of events is factually bulletproof; surely it is not.[33] Given the factual particulars alleged, however, the inference of scienter is "at least as compelling" as an inference non-fraudulent intent, which means that these allegations survive the PSLRA standard, and should go forward to the discovery phase and subjected to proof or disproof.

3. Section 20(a) claims

Defendants argue that the plaintiffs' "control person" liability claim must fail for failure to plead an underlying violation of the Exchange Act. I have already found, however, that such a violation has been adequately pled. The motion to dismiss the section 20(a) claim against Peltz or Kovacs is therefore

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NDA because the application failed to meet minimal FDA review standards (*e.g.*, that the application contain *at least some* pre-specified data evincing efficacy in more than one clinical trial), not because of some secret FDA requirement. PTC, in other words, allegedly knew that the "lack of statistically significant results in the ITT population . . . would be the device's downfall or that such a lack was so obvious an impediment" to the 2016 NDA that PTC's "failure to perceive the risk of non-approval was reckless . . . ." *Id.* at 44. The issue is concededly a debatable one, but for the reasons stated herein, I think the AC alleges sufficient facts to raise an inference of scienter that is at "least as compelling as any" inference of non-fraudulent intent.

[33] Among the allegations that will be tested in discovery is the assertion that certain manipulations of clinical data were not honestly believed or lacked a reasonable basis, or were performed to cover up known deficiencies in the clinical data, or did not conform to FDA guidance.

dismissed on this basis. I make no ruling as any other issue, such as whether the AC pleads "circumstances establishing the defendant's control over the company's actions." *The Winer Family Trust v. Queen*, No. Civ.A. 03-4316, 2004 WL 2203709, at *22 (E.D. Pa. Sept. 27, 2004).

III. CONCLUSION

For the reasons set forth above, the defendants' motion to dismiss is GRANTED IN PART AND DENIED IN PART. Specifically, the motion to dismiss is GRANTED as to PTC's statements about Translarna's FDA review timeline (AC ¶¶ 108-112) and the risk of the ACT DMD study (*id.* ¶¶ 113-125), but DENIED as to PTC's statements about the ACT DMD results. (*Id* ¶¶ 126-155).

For the reasons stated in I.C.1 n.5, plaintiffs' motion to strike ECF Nos. 56-21, 56-22, 56-30–56-33, 56-35, and 56-36, is GRANTED. In all other respects, the motion is DENIED.

A separate order will issue.

Dated: August 28, 2017

KEVIN MCNULTY
United States District Judge